Spina Bifida: A Review of the Genetics, Pathophysiology and Emerging Cellular Therapies.

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Public Summary:
Spina bifida, the most common congenital central nervous system defect, results from incomplete closure of the spinal canal during development. The physical and chemical trauma resulting from exposure to the fetal spinal cord causes impaired lower extremity, bowel and bladder function. Though the causes of this disease are not fully understood, many great advancements in research have been achieved regarding treatment options due to research that has better characterized the molecular processes associated with spina bifida. This review covers the advancements in genetics, embryology, and pathophysiology research of spina bifida, and concludes with a discussion of current clinical trials testing spina bifida therapies. Furthermore, this review summarizes the progression of postnatal surgical correction to stem cell therapeutic based augmentation of in utero spina bifida repair and the beginning of the first FDA-approved clinical trial testing the efficacy of stem cell-based therapies for prenatal spina bifida treatment.

Scientific Abstract:
Spina bifida is the most common congenital defect of the central nervous system which can portend lifelong disability to those afflicted. While the complete underpinnings of this disease are yet to be fully understood, there have been great advances in the genetic and molecular underpinnings of this disease. Moreover, the treatment for spina bifida has made great advancements, from surgical closure of the defect after birth to the now state-of-the-art intrauterine repair. This review will touch upon the genetics, embryology, and pathophysiology and conclude with a discussion on current therapy, as well as the first FDA-approved clinical trial utilizing stem cells as treatment for spina bifida.